Stopping TSC Onset and Progression 2B: Sirolimus TSC Epilepsy Prevention Study

Status: RECRUITING

Eligibility Criteria

Sex: ALL
Age: 1 day to 6 months old
Healthy Volunteers: This study is NOT accepting healthy volunteers
Inclusion Criteria:

1. 0-6 months of age at the time of enrollment (subject must be <7 months of chronological age at time of randomization and treatment initiation). Corrected age must be at least 39 weeks (calculated by subtracting the number of weeks born before 40 weeks gestation from the chronological age).
2. Has a confirmed diagnosis of TSC based on established clinical or genetic criteria

Exclusion Criteria:

1. Prior history of seizures (clinical or electrographic) at the time of enrollment or identified on baseline EEG.
2. Has been treated in the past or is currently being treated at the time of enrollment with conventional anticonvulsant medications (AEDs), systemic (oral) mTOR inhibitors (such as rapamycin, sirolimus, or everolimus), ketogenic-related special diet, or another anti-seizure therapeutic agent, device, or procedure.
3. Has taken any other investigational drug as part of another research study, within 30 days prior to the baseline screening visit.
4. Has a significant illness or active infection at the time of the baseline screening visit
5. Has a history of significant prematurity, defined as gestational age <30 weeks at the time of delivery, or other significant medical complications at birth or during the neonatal period that other than TSC would convey additional risk of seizures or neurodevelopmental delay (i.e. HIE, severe neonatal infection, major surgery, prolonged ventilatory or other life-saving supportive care or procedures).
6. Abnormal laboratory values at baseline (i.e., renal function, liver function, or bone marrow production) that are in the opinion of the investigator clinically significant and may jeopardize the safety of the study subject.
7. Prior, planned or anticipated neurosurgery within 3 months of the baseline visit
8. Has a TSC-associated condition for which mTOR treatment is clinically indicated (i.e. SEGA or AML).
9. Subjects who are, in the opinion of the investigator, unable to comply with the requirements of the study.

Conditions & Interventions

Interventions:
DRUG: Sirolimus, DRUG: Placebo
Conditions:
Tuberous Sclerosis Complex, Epilepsy
Keywords:
Tuberous Sclerosis Complex, TSC, epilepsy, prevention, mTOR, sirolimus, infant

More Information

Description:
This trial is a Phase II randomized, double-blind, placebo controlled multi-site study to evaluate the safety and efficacy of early sirolimus to prevent or delay seizure onset in TSC infants. This study is supported by research funding from the Office of Orphan Products Division (OOPD) of the US Food and Drug Administration (FDA).

Contact(s): Molly Griffith - Molly.Griffith@cchmc.org
Principal Investigator:
Principal Investigator ID:
Phase: PHASE2
IRB Number:
System ID: NCT05104983